

# PATENT COOPERATION TREATY

## PCT

### INTERNATIONAL SEARCH REPORT

(PCT Article 18 and Rules 43 and 44)

Applicant's or agent's file reference 10589-34-228	<b>FOR FURTHER ACTION</b> see Form PCT/ISA/220 as well as, where applicable, item 5 below.	
International application No. PCT/US04/09590	International filing date ( <i>day/month/year</i> ) 26 March 2004 (26.03.2004)	(Earliest) Priority Date ( <i>day/month/year</i> ) 27 March 2003 (27.03.2003)
Applicant PTC THERAPEUTICS, INC.		

This international search report has been prepared by this International Searching Authority and is transmitted to the applicant according to Article 18. A copy is being transmitted to the International Bureau.

This international search report consists of a total of 5 sheets.



It is also accompanied by a copy of each prior art document cited in this report.

**1. Basis of the Report**

- a. With regard to the language, the international search was carried out on the basis of the international application in the language in which it was filed, unless otherwise indicated under this item.

☐

The international search was carried out on the basis of a translation of the international application furnished to this Authority (Rule 23.1(b)).

- b. ☐ With regard to any nucleotide and/or amino acid sequence disclosed in the international application, see Box No. I.

2. ☒ Certain claims were found unsearchable (See Box No. II)

3. ☒ Unity of invention is lacking (See Box No. III)

4. With regard to the title,

☒

the text is approved as submitted by the applicant.

☐

the text has been established by this Authority to read as follows:

5. With regard to the abstract,

☒

the text is approved as submitted by the applicant.

☐

the text has been established, according to Rule 38.2(b), by this Authority as it appears in Box No. IV. The applicant may, within one month from the date of mailing of this international search report, submit comments to this Authority.

6. With regard to the drawings,

- a. the figure of the drawings to be published with the abstract is Figure No. \_\_\_\_\_

☐

as suggested by the applicant.

☐

as selected by this Authority, because the applicant failed to suggest a figure.

☐

as selected by this Authority, because this figure better characterizes the invention.

- b. ☒ none of the figures is to be published with the abstract.

# INTERNATIONAL SEARCH REPORT

International application No.

PCT/US04/09590

## A. CLASSIFICATION OF SUBJECT MATTER

IPC(7) : A01N 61/00; C12Q 1/00; G01N 33/566, 33/573, 33/574

US CL : 435/ 4, 6, 7.2, 7.21, 41, 69.2, 91.3, 183 ; 514/ 1, 2

According to International Patent Classification (IPC) or to both national classification and IPC

## B. FIELDS SEARCHED

Minimum documentation searched (classification system followed by classification symbols)

U.S. : 435/ 4, 6, 7.2, 7.21, 41, 69.2, 91.3, 183 ; 514/ 1, 2

Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched

Electronic data base consulted during the international search (name of data base and, where practicable, search terms used)  
Please See Continuation Sheet

## C. DOCUMENTS CONSIDERED TO BE RELEVANT

Category *	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
X	US 5,726,195A (HILL et al.) 10 March 1998, see entire patent.	18-19
X	US 6,446,032 B1 (SCHIMMEL) 03 September 2002, see entire patent document.	20-21
X	WO 02/083837 A1 (PTC CORPORATION, INC.) 24 October 2002, see entire document, especially abstract, examples and claims.	18-21
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Y		1-24
X	WO 02/083953 A1 (PTC CORPORATION, INC.) 24 October 2002, see entire document, especially abstract, examples and claims.	18-21
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Y		1-24
X	WO 01/25486 A1 (UNIVERSITY OF MEDICINE AND DENTISTRY OF NEW JERSEY) 12 April 2001, see entire document, especially abstract, examples and claims.	18-21
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Y		1-24



Further documents are listed in the continuation of Box C.



See patent family annex.

\* Special categories of cited documents:

"A" document defining the general state of the art which is not considered to be of particular relevance

"E" earlier application or patent published on or after the international filing date

"L" document which may throw doubts on priority claim(s) or which is cited to establish the publication date of another citation or other special reason (as specified)

"O" document referring to an oral disclosure, use, exhibition or other means

"P" document published prior to the international filing date but later than the priority date claimed

"T"

later document published after the international filing date or priority date and not in conflict with the application but cited to understand the principle or theory underlying the invention

"X"

document of particular relevance; the claimed invention cannot be considered novel or cannot be considered to involve an inventive step when the document is taken alone

"Y"

document of particular relevance; the claimed invention cannot be considered to involve an inventive step when the document is combined with one or more other such documents, such combination being obvious to a person skilled in the art

"&"

document member of the same patent family

Date of the actual completion of the international search

15 December 2004 (15.12.2004)

Date of mailing of the international search report

27 JAN 2005

Name and mailing address of the ISA/US

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# INTERNATIONAL SEARCH REPORT

International application No.  
PCT/US04/09590

## C. (Continuation) DOCUMENTS CONSIDERED TO BE RELEVANT

Category *	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
X --- Y	GREER, C.L. Assembly of a tRNA Splicing Complex: Evidence for Concerted Excision and Joining Steps in Splicing In Vitro. Molecular and Cellular Biology. February 1986, Vol. 6, No. 2, pages 635-644, see entire article, especially Fig. 1, pages 638-642.	1, 7, 8, 11, 13, 15 and 17 ----- 1-24
Y	LI et al. Crystal Structure and Evolution of a Transfer RNA Splicing Enzyme. Science. 10 April 1998, Vol. 280, No.10, pages 279-284, see entire article.	1-24
Y	HYDE-DERUYSCHE et al.. Detection of Small-Molecule Enzyme Inhibitors With Peptides Isolated From Phage-Displayed Combinatorial Peptide Libraries. Chemistry & Biology. 2000, Vol. 7, No. 1, pages 17-25, see entire article, especially Abstract, Table 1 and pages 23-24.	1-24
A	ABELSON et al. tRNA Splicing. J. Biol. Chem. 22 May 1998, Vol. 273, No. 21, pages 12685-12688, see entire article.	1-24
A	TROTTA et al. The Yeast tRNA Splicing Endonuclease: A Tetrameric Enzyme With Two Active Site Subunits Homologous To The Archaeal tRNA Endonucleases. Cell. 13 June 1997, Vol. 89, pages 849-858, see entire article.	1-24
A	VAUGHAN et al. Methionine In and Out of Proteins: Targets for Drug Design. Current Medicinal Chemistry. 2002, Vol. 9, No. 3, pages 385-409, see entire article.	1-24

# INTERNATIONAL SEARCH REPORT

International application No.

PCT/US04/09590

## BOX III. OBSERVATIONS WHERE UNITY OF INVENTION IS LACKING

This application contains the following inventions or groups of inventions which are not so linked as to form a single general inventive concept under PCT Rule 13.1. In order for all inventions to be examined, the appropriate additional examination fees must be paid.

Group I, claim(s) 1-3, 7(in part), 8, 11-14(in part), 15, 17(in part), 22 and 23-24(in part), drawn to assaying for an antifungal compound that inhibits fungal tRNA splicing ligase.

Group II, claim(s) 4-6, 7(in part), 9-10, 11-14(in part), 16, 17(in part) and 23-24 (in part) drawn to assaying for an anti-proliferative compound that inhibits animal tRNA splicing ligase.

Group III, claim(s) 18-19, drawn to preventing/treating a fungal infection by administering an antifungal compound identified by the Group I method.

Group IV, claim(s) 20-21, drawn to treating a proliferative disorder by administering an anti-proliferative compound identified by the Group II method.

The inventions listed as Groups I-IV do not relate to a single general inventive concept under PCT Rule 13.1 because, under PCT Rule 13.2, they lack the same or corresponding special technical features for the following reasons: the methods of Groups I and II are distinctly different methods drawn to different method objectives and utilize different sources of enzymes. The antifungal and antiproliferative compounds of Groups III and IV derived from the Group I and II methods do not represent a "special" technical feature since antifungal and antiproliferative compounds are known in the art. See e.g. see RNA binding "small molecule" compounds disclosed in WO 02/083953A1; WO 02/083837A1; and WO 01/25486A1.

Continuation of B. FIELDS SEARCHED Item 3:  
WEST: PGPB, USPT, USOC, EPAB, JPAB, DWPI, TDBD.  
STN: CAPLUS, EMBASE, BIOSIS, MEDLINE, WPIDS.